hNSC-mediated delivery of ApiCCT1 as a candidate therapeutic for Huntington's disease

Grant Award Details

hNSC-mediated delivery of ApiCCT1 as a candidate therapeutic for Huntington's disease

Grant Type: Quest - Discovery Stage Research Projects

Grant Number: DISC2-09569

Project Objective: Evaluate the synergistic therapeutic benefit of combined hNSC (ESI-017) transplantation with continuous secretion of ApiCCT1 resulting in demonstrated efficacy for the candidate cell therapy.

Investigator:

Name:	Leslie Thompson
Institution:	University of California, Irvine
Type:	PI

Disease Focus: Huntington's Disease, Neurological Disorders

Human Stem Cell Use: Embryonic Stem Cell

Award Value: $1,650,263

Status: Active

Grant Application Details

Application Title: hNSC-mediated delivery of ApiCCT1 as a candidate therapeutic for Huntington's disease

Public Abstract:

Research Objective

The therapeutic candidate is a human Neural Stem Cell that secretes a protein, ApiCCT1, that aids in the prevention of disease phenotypes, for application in treatment of Huntington's disease (HD).

Impact

No treatment currently exists that can slow or prevent the unrelenting progression of Huntington's disease, a devastating brain disease, therefore a completely unmet medical need exists.

Major Proposed Activities

- Generation of a Good Manufacturing Practice (GMP) grade lentivirus to deliver ApiCCT1 to stem cells.
- Generation of a quality controlled bank of GMP grade human Neural Stem cells (hNSCs) that express a secreted form of the molecular therapeutic (ApiCCT1) and characterization of ApiCCT1 expression.
- Test delivery of ApiCCT1 expressing hNSCs to the striatum of Huntington's disease mouse model and determine whether this stem cell candidate can provide neuroprotection.
- Manufacture of a GMP grade human embryonic stem cell (hESC) bank with ApiCCT1 integrated into the genome at a safe harbor site. Cells will be characterized and expanded.
- Test delivery of hESC-derived hNSCs expressing secreted ApiCCT1 to the striatum of rapidly progressing HD mice and determine whether this stem cell candidate can provide neuroprotection.
- Test delivery of hESC-derived hNSCs expressing secreted ApiCCT1 to the striatum of slower progressing HD mice and determine whether this stem cell candidate can provide neuroprotection.

Statement of Benefit to California:

The disability, loss of personal freedom and earning potential, and costly institutional care of Huntington's disease (HD) is devastating. Developing a candidate therapeutic for HD will benefit the State through new technologies and intellectual property resulting in possible job creation and revenues in new companies, in addition to the potential for substantial reductions in individual suffering, medical and care-giving costs.
